Rationale for Therapy Discontinuation in Patients With Lower-Risk Transfusion-Dependent Myelodysplastic Syndromes (MDS)

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INTRODUCTION

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METHODS

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METHODS

Study Design

Physicians were recruited via e-mail from a U.S. nationally representative online panel of hospital-based hematologists and physicians who are active in patient care in multiple research settings

The inclusion of panel members was not based on consensus, as an intent was made to match the characteristics of the broader specialty population of the American Medical Association

- Participating physicians were compensated for their participation

The study protocol was HRA approved

Sample

- Data were derived from disease-specific physician surveys and patient charts, which provided information on demographics, treatments, and outcomes in TD lower-risk MDS patients

- Patients with MDS who received ≥1 therapy for ≥24 months, with varying disease history and treatment-transfusion dependency (TD), were investigated in this study

- In addition to transfusions, treatments to improve hemoglobin levels have historically been limited to erythropoiesis-stimulating agents (ESAs), whereas patients with more advanced disease may require treatment with hypomethylating or targeted chemotherapeutic agents associated with platelet or leukocyte reduction (TD)

- As there is no standard treatment course, these therapies are continued until unacceptable toxicity, lack of response, or disease progression

OBJECTIVE

- To examine treatment patterns, clinical outcomes, and the physician’s report of reason for treatment discontinuation (previously shown to vary from patients’ perspectives) in patients who become TD at or after MDS diagnosis

RESULTS

The majority of patients received only 1 therapy for MDS during the study period

- Patients who received more than 1 therapy often received ESA first, but in some cases treatments overlapped

- The median time to discontinuation of 13 months

- The majority of patients received both LDHs and HMAs

- 35% of patients received both ESAs and HMAs

- The model included terms for the potential interaction of disease stage with the primary outcome of achieving transfusion independence (TI)

- This was true for each of the treatment groups as well (Figure 4)

- The number of patients who stop therapy for alternative reasons suggests that the majority of patients do not require management of transfusion dependency, which may have introduced recall bias related to those data points were included

- For patients who stop therapy for alternative reasons, Ti was defined as the time from diagnosis at which the majority of patients in that group (90%) were transfusion independent or transfusion expectation (Figure 3)

- The number of patients who stop therapy for alternative reasons suggests that the majority of patients do not require management of transfusion dependency, which may have introduced recall bias related to those data points were included

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- As there is no standard treatment course, these therapies are continued until unacceptable toxicity, lack of response, or disease progression

Figure 1. Treatment Usage in Addition to RBC Transfusion Among Patients With TD Lower-Risk MDS

Figure 2. Reasons for MDS Treatment Discontinuation by Therapy

Figure 3. Reasons for MDS Treatment Discontinuation by Therapy

Figure 4. Reasons for MDS Treatment Discontinuation By Therapy

Figure 5. Reasons for MDS Treatment Discontinuation by Therapy

Table. Demographics, Health Characteristics, and Disease History of Patients With Lower-Risk MDS

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>Total Sample (N = 1,221)</th>
<th>TD Low/Intermediate-1-risk MDS</th>
<th>TD Lower-Risk MDS</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age, median (range)</td>
<td>65 years (27–95)</td>
<td>64.9 ± 12.0</td>
<td>64.9 ± 12.0</td>
</tr>
<tr>
<td>Gender, Male, n (%)</td>
<td>690 (56.3)</td>
<td>417 (52.3)</td>
<td>273 (55.2)</td>
</tr>
<tr>
<td>IPSS risk at diagnosis, n (%)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>+3 abnormalities</td>
<td>235 (19.3)</td>
<td>164 (20.8)</td>
<td>71 (14.6)</td>
</tr>
<tr>
<td>+2 abnormalities</td>
<td>20 (1.6)</td>
<td>14 (1.8)</td>
<td>6 (1.2)</td>
</tr>
<tr>
<td>+1 abnormalities</td>
<td>9 (0.7)</td>
<td>5 (0.6)</td>
<td>4 (0.8)</td>
</tr>
<tr>
<td>Complex, &gt; 3 abnormalities</td>
<td>360 (29.5)</td>
<td>221 (28.1)</td>
<td>139 (27.7)</td>
</tr>
<tr>
<td>Known</td>
<td>969 (79.5)</td>
<td>626 (79.4)</td>
<td>343 (69.7)</td>
</tr>
<tr>
<td>Unknown</td>
<td>232 (19.1)</td>
<td>195 (24.7)</td>
<td>37 (7.4)</td>
</tr>
</tbody>
</table>
| Median time to discontinuation of 13 months

DISCUSSION

The number of patients who stop therapy for alternative reasons suggests that the majority of patients do not require management of transfusion dependency, which may have introduced recall bias related to those data points were included

CONCLUSIONS

- The number of patients who stop therapy for alternative reasons suggests that the majority of patients do not require management of transfusion dependency, which may have introduced recall bias related to those data points were included

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REFERENCES


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DISCLOSURES

A.T.G. receives research support from Amgen, Celgene, and Janssen (J&J) for studies that were sponsored by these companies. G.B., M.M.: employees of Celgene Corporation. M.A.S.: is a consultant and receives research support from Celgene Corporation for work unrelated to this poster. F.J. is a member of the Advisory Board of Board of Directors of Celgene and receives research support in support of Celgene-sponsored studies as a consultant and receives research support in support of Celgene-sponsored studies as a consultant. H.C.: Basic Research-Experimental Medicine, Editorial Board of Excerpta Medica. Resume and research support in support of Celgene-sponsored studies as a consultant and receives research support in support of Celgene-sponsored studies as a consultant. H.C.: Basic Research-Experimental Medicine, Editorial Board of Excerpta Medica. Resume and research support in support of Celgene-sponsored studies as a consultant and receives research support in support of Celgene-sponsored studies as a consultant.